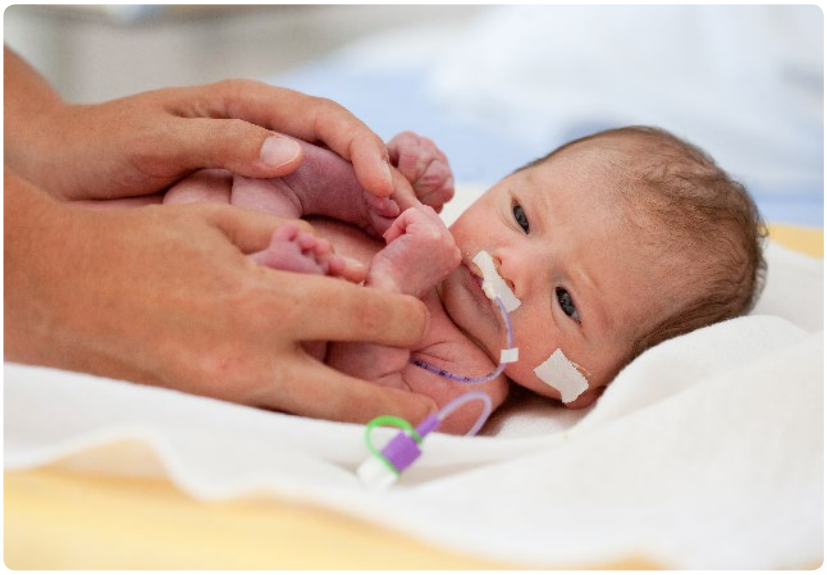
Shoring Up the Still-Emerging Science of Neonatology



Above: A pediatrician puts a premature newborn baby into a fetal position to help him relax. Right: Neonatologist and Lead Medical Officer Gerri Baer is a welcome new face at FDA, focusing on drug and device product development, safety, and labeling for neonates.

Neonatologist Gerri Baer is a welcome new face at FDA. Since she began at the agency in September 2015, she has delved deeply into her new role, focusing on drug and device product development, safety, and labeling for neonates. Consumer Updates asked her to talk about the work she will be tackling at FDA.

Q: How do we define "neonate" and "neonatology"?

A: Neonates are a very unique population. A neonate can weigh 1 pound or 11 pounds, so even within the "neonate" category, there is tremendous variation. Full-term neonates are born between 37 and 41 weeks' gestation, and are considered neonates for their first 28 days of life. Premature infants should be considered "neonates" until they are 4 weeks beyond their due date. So an infant born at 24 weeks' gestation would be defined as a neonate until she reaches 40 weeks' gestation, plus 28 days.

It's helpful to understand that many of a pre-term infant's organs are immature at birth. A lot of maturation that normally would have been done in-utero—where the mother is essentially the baby's life support system—now has to go on outside the mother.

Q: What motivated you to accept this new role at FDA?

A: I love providing clinical care to neonates, but one of my long-standing concerns has been the number of treatments we use off-label. This means a drug is used in a way that is different from that described in the FDA-approved drug label. Many off-label medications have been used in the NICU for decades, and as a result,

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it is challenging to conduct randomized trials. In adult drug development, randomized, controlled clinical trials are the gold standard for approval and labeling. In neonatal development, alternative methods of assessing a treatment's safety and efficacy may be appropriate.

From my work as a practicing clinician, I am also aware of the difficulties in performing high-quality trials and rigorous safety monitoring of therapies in neonates. The history of neonatology was marked by treatments that appeared beneficial, but turned out to have problematic long-term effects, such as oxygen and dexamethasone for babies with respiratory distress syndrome.

There have been major regulatory improvements, and pharmaceutical companies now are required either to study all new therapies in children or justify not studying them. There are a number of conditions that occur only in this population, and I want to bring more regulatory attention to neonatal therapeutics.

Q: What role does FDA play in the world of neonatology, and how will you contribute to that role?

A: FDA improves and protects the health of the American public, including our smallest citizens. The agency is responsible for deciding which drugs and devices may be labeled for use and marketed to the public.

I consult with all of the centers at FDA with respect to neonatal issues, including trial design, feasibility, current standards of care, and neonatal ethics. I bring a clinical perspective and knowledge base to this highly specialized area.

Q: What are you looking forward to accomplishing?

A: One of my core goals is to protect the tiniest and some of the most vulnerable members of our population!

Since I am charged with developing a comprehensive neonatology program at FDA, I'm in the process of assessing the needs of both the agency and external stakeholders such as the industry and the academic communities, to figure out what that means and what needs to be done.

In addition, I want to draw more attention internally at FDA to the therapeutic challenges in neonatology, and to work within the agency in any way possible to encourage development of neonatal therapies.

I'm particularly interested in enhancing our ability to monitor for adverse drug or device events in babies. I'm going to be focusing in part on learning about what kind of safety signals we might receive and what safety signals we might be missing that we could find a way to collect.

Q: How does FDA use information on adverse events? Why is it important to shore up that data?

A: In my years in clinical practice, I do not remember a single instance where I reported an adverse drug or device event to the FDA. I cannot recall any training about adverse event reporting outside the hospital, and I do not believe I am unique in this regard. Clinicians working with neonates are on the front line of our understanding of drug and device safety, and their input is crucial.

For example, if a small IV catheter used in neonates breaks once or twice a year in your NICU, you may not think much of it, but if there are 50 or 100 such events in a year across the country, it is something FDA needs to be aware of, so that the Agency can get the word out to the manufacturer and to the practitioners using the product. Unless FDA receives reports on adverse events, there may not be recognition that a product has safety issues. My goal is to make the world a safer place for some of our most vulnerable citizens. Strengthening detection and improving the response to problems with FDA-regulated products is part of the Agency's core mission goals and objectives. FDA

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